



Muscular Dystrophy
New Zealand

SMA Community Newsletter

Update on SMA Treatments

Starship Children's Health is able to offer nusinersen treatment to infants with Type 1 Spinal Muscular Atrophy (SMA), though an Expanded Access Programme sponsored by Biogen. Nusinersen is the first treatment for SMA to be approved by the American FDA. It is not yet registered or funded for use in New Zealand. The Expanded Access Programme is being offered by Biogen as an unregistered medicine, to meet an unmet clinical need for severely affected children with Type 1 SMA, whilst Medsafe and PHARMAC consider an application for registration and funding, respectively. Type 1 Spinal Muscular Atrophy is a devastating neurological disease and the most common genetic cause of infantile death. Affected infants present before 6 months of age with muscle weakness and never

achieve the ability to sit. With no treatment options available, infants develop respiratory failure and historically die before 2 years of age. Nusinersen is administered 4 monthly as an intrathecal injection, and works to increase levels of the defective protein. Treatment has been proven to improve survival and muscle strength in treated infants. The best outcomes are seen with early initiation of treatment. Eligibility for access to the programme will be determined by the National Nusinersen Review Committee comprising medical and community input. Enquiries should be addressed to the Paediatric Neuroservices Department at Starship Hospital - PaedNeuro@adhb.govt.nz. We hope that Nusinersen will be able to be offered to children with other types of SMA. Unfortunately, there is not currently any access to

this treatment in New Zealand for children with Type 2 or 3 SMA. Biogen will be making an application to PHARMAC for funding. A PHARMAC decision regarding this application is not anticipated until 2019.

SMA FAMILY DAY

RSVP Now!

Saturday 22nd
September 2018,
11am-4.00pm,

MDANZ National Office,
419 Church Street East,
Penrose, Auckland.

Join us for lunch and
hear from expert
speakers.

Meet new families and
ask questions from our
panel of experts.

Travel assistance is
available.

RSVP info@mda.org.nz
or call 0800 800 337

SMA Stories

Created by the team at MDANZ, a new book aims to raise awareness of SMA. The launch of SMA Stories will take place on the 28th August more information to come.

Cure SMA Conference in Dallas, Texas

Fiona Tolich and Anna Sutherland (pictured below) attended the Cure SMA Conference in June. As the only Kiwis at the conference, the pair decided to split up and attend different workshops to cover more of the conference. "When they say everything is bigger in Texas they mean it. The conference was no different. There were over 55 family workshop sessions and 185 research and care presentations to choose from, the toughest part was deciding which to attend. Anna focused on sessions relevant for parents of children with SMA and picked up some valuable practical hints from being at the workshops.

Things like putting a rubber glove on her child's hand to create resistance when he is in the pool. "Just little things that I would never have thought of," says Anna. Many of the attendees at the conference access Spinraza and seeing this fueled Fiona's passion to continue advocating for everyone New Zealander to have the access. To read detailed reports of the conference visit the [MDANZ website](http://MDANZwebsite.com). Next year's conference will take place in Anaheim, California – Disneyland!



Fiona Tolich and Anna Sutherland at the Cure SMA Conference in Texas.

SMA Member Reference Group

The Group met again on the 24th July 2018. Plans are well underway for the September SMA Family Day. Importantly, a template letter, raising awareness of SMA and the new treatment, for people to send to their MP was reviewed and signed off by the group. The template is available as a MS Word document on MDANZ's [click here for template](#) for you, your family and friends to personalise and send to your Member of Parliament. Reference group members are pictured below; Tania Woodmass, Brent Walker, Emily Beswick, Fiona Tolich, Jo Truscott, Belinda Old and Anna Sutherland.



The SMA Reference Group meeting at the MDA Office in Auckland.